16 October 2017
EMA/476085/2017

Public summary of opinion on orphan designation

Odiparcil for the treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome)

On 23 August 2017, orphan designation (EU/3/17/1903) was granted by the European Commission to Inventiva, France, for odiparcil for the treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome).

What is mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome)?

Mucopolysaccharidosis type VI (also known as Maroteaux-Lamy syndrome) is an inherited disease that is caused by the lack of an enzyme called arylsulfatase B (ARSB). This enzyme is needed to break down substances in the body called glycosaminoglycans (GAGs). If the enzyme is not present, GAGs cannot be broken down and they build up in the cells and damage them. This causes a wide range of symptoms, the most noticeable being a short body, a large head, difficulty moving about, clouding of the eyes and hearing loss. The disease is usually diagnosed in children between one and five years of age.

Mucopolysaccharidosis VI is a long-lasting and life-threatening disease because of the damage to various parts of the body, particularly spine, heart and lungs.

What is the estimated number of patients affected by the condition?

At the time of designation, mucopolysaccharidosis VI affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, galsulfase was authorised in the EU for the treatment of mucopolysaccharidosis VI. This is an 'enzyme replacement therapy' which works by providing patients

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

with the enzyme they are lacking. Some patients underwent transplantation to receive haematopoietic (blood) stem cells that are able to produce the missing enzyme.

The sponsor has provided sufficient information to show that odiparcil might be of significant benefit for patients with mucopolysaccharidosis VI. Laboratory studies indicate that odiparcil may improve symptoms not managed by the currently authorised medicine, such as clouding of the eyes. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Odiparcil works by attaching to an enzyme called galactosyl transferase I, which is responsible for starting production of GAGs. By attaching to this enzyme, odiparcil is expected to reduce production of GAGs, thereby relieving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of odiparcil have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with odiparcil in patients with mucopolysaccharidosis VI were ongoing.

At the time of submission, odiparcil was not authorised anywhere in the EU for mucopolysaccharidosis VI or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2017 recommending the granting of this designation.

_____

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Odiparcil	Treatment of mucopolysaccharidosis type VI (Maroteaux-Lamy syndrome)
Bulgarian	Одипарцил	Лечение на Мукополизахаридоза тип VI (Maroteaux-Lamy syndrome)
Croatian	Odiparcil	Liječenje mukopolisaharidoze tipa VI (Maroteaux-Lamy)
Czech	Odiparcil	Léčba mukopolysacharidosy typu VI (Maroteaux-Lamy syndrom)
Danish	Odiparcil	Behandling af mukopolysakkaridose type VI (Maroteaux-Lamy syndrom)
Dutch	Odiparcil	Behandeling van mucopolysaccharidosis, type VI (Maroteaux-Lamy syndroom)
Estonian	Odipartsiill	Mukopolüsahharidoos tüüp VI (Maroteaux-Lamy sündroom) ravi
Finnish	Odiparsiili	Mukopolysakkaridoosi VI:n (Maroteaux-Lamy-syndrooma) hoito
French	Odiparcil	Traitement de mucopolysaccharidose, type VI (syndrome de Maroteaux et Lamy)
German	Odiparcil	Behandlung der Mukopolysaccharidose Typ VI (Maroteaux-Lamy-Syndrom)
Greek	Οντιπαρσίλη	Θεραπεία της βλεννοπολυσακχαρίδωσης τυπου VI (σύνδρομο Maroteaux-Lamy)
Hungarian	Odiparcil	VI-típusú mucopolysaccharidosis (Maroteaux-Lamy szindróma) kezelèse
Italian	Odiparcil	Trattamento della mucopolisaccaridosi tipo VI (sindrome di Maroteaux-Lamy)
Latvian	Odiparcils	VI tipa mukopolisaharidozes (Marota-Lamī (*Marateux-Lamy*) sindroma) ārstēšana
Lithuanian	Odiparcilis	VI tipo mukopolisacharidozės (*Maroteaux-Lamy* sindromo) gydymas
Maltese	Odiparsil	Kura tal-mukopolisakkaridożi tat-tip VI (sindrome ta' Maroteaux-Lamy)
Polish	Odiparcyl	Leczenie mukopolisacharydozy typu VI (zespół Maroteaux-Lamy)
Portuguese	Odiparcil	Tratamento da Mucopolissacaridose tipo VI (síndrome de Maroteaux-Lamy)
Romanian	Odiparcil	Tratamentul mucopolizaharidozei tip VI (sindromul Maroteaux-Lamy)
Slovak	Odiparcil	Liečba mukopolysacharidózy typu VI (Maroteauxov-Lamyho syndróm)
Slovenian	Odiparcil	Zdravljenje mukoplisaharidoze tipa VI (Maroteaux-Lamyov sindrom)
Spanish	Odiparcil	Tratamiento de la Mucopolisacaridosis tipo VI (síndrome de Maroteaux-Lamy)
Swedish	Odiparcil	Behandling av mukopolysackaridos typ VI (Maroteaux-Lamy syndrom)
Norwegian	Odiparcil	Behandling av mukopolysakkaridose type VI (Maroteaux-Lamy syndrom)
Icelandic	Ódiparíil	Meðferð á múkópólýsakkarídósis tegund VI (Maroteaux-Lamy heilkenni)

[1] At the time of designation